observed for age, pharmacologic treatment and disease severity. No significant statistical effect was observed for gender, late onset patients (>50 years) and liver transplant. **CONCLUSIONS:** The preference-based utility measures used in this study adequately disentangle TTR-FAP impact on patient's quality of life and allow discriminating across different TTR-FAP clinical severity states, interventions and demographic characteristics. Assuming that these values represent the patients' preferences and the utility associated with their health state, the results presented in this study may be used in future health technologies cost-utility studies.

PSY98

HEALTH-RELATED QUALITY OF LIFE (HRQOL) IN SPLENECTOMIZED IMMUNE THROMBOCYTOPENIA (ITP) PATIENTS – A TARGETED LITERATURE REVIEW

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OBJECTIVES: Immune thrombocytopenia (ITP) is associated with a risk of spontaneous and excessive bleeding. Among the treatment options are medical therapies and splenectomy – a major intervention with lifelong consequences for the patient. Both ITP and ITP-treatment may impact health-related quality of life (HrQoL); therefore, a literature search was conducted to assess HrQoL of splenectomized compared to non-splenectomized ITP patients. **METHODS:** A targeted literature search was conducted via Ovid (Medline, Embase, and Cochrane) in September 2014. Search strings were based on PICO-criteria (Patient, Intervention, Comparison and Outcomes). Inclusion criteria were: studies of adult patients with ITP that contained information on HrQoL; clinical trials, RCTs, meta-analyses, observational trials, retrospective studies or systematic reviews; manuscripts and congress abstracts in English or German language. **RESULTS:** The search identified 148 potentially relevant publications. After removing duplicates, 120 titles and abstracts were screened. Following the title/abstract screening, a full text screening (n=21) identified six relevant publications. HrQoL was assessed in these six publications using generic (SF-36, n=2; EQ-5D, n=1) and disease-specifc (ITP-PAQ, n=4) instruments. Two studies using generic instruments reported no difference in HrQoL between splenectomized and non-splenectomized patients. Of the four studies using the ITP-PAQ, two reported no difference and two found worse HrQoL in splenectomized versus non-splenectomized patients. In the only study that included both generic (SF-36, EQ-5D) and ITP-specific measures, results were inconsistent, as they varied in outcomes. Overall detailed results were not always provided and HrQoL was not reported according to response to therapy in any study. **CONCLUSIONS:** The impact of splenectomy on HrQoL in patients with ITP is inconclusive and inconsistently reported in the literature, due to use of different instruments, diverse patient characteristics, and pooling of data among responding and non-responding patients. Further studies are needed to address this question within clearly-defined populations.

PSY99

ASSESSING THE TREATMENT BURDEN FOR GROWTH HORMONE DEFICIENCY (GHD) IN CHILDREN: CONCEPT ELICITATION RESULTS SUPPORTING THE DEVELOPMENT OF THE TREATMENT BURDEN MEASURE FOR CHILDHOOD GHD (TB-CGHD)

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OBJECTIVES: Treatment for children with GHD involves daily injections which may begin at a very early age and continue throughout childhood. However, the treatment burden (TB) for children and their parents has not been examined. The purpose of this study was to support the content validity of a new GHD-specific TB measure, with versions for patient-reported outcome (PRO) for older children, and observer-reported outcome (ObsRO) and PRO for parents/guardians. **METHODS:** Focus groups and interviews were conducted with 39 children (aged 8–12) with GHD, 31 parents of children (aged 4–12) and eight clinical experts in three countries (Germany, UK, US). Interviews were analysed and coded using adapted grounded theory. A conceptual model of TB was developed and items were generated and then cognitively debriefed. **RESULTS:** Qualitative analysis found saturation was reached with three domains for child TB and two domains for parent TB. Child domains (and major proximal subdomains) were: Physical (pain, 33% and bruising, 19%), Interference (Interference with overnight or other activities, 29% and Time needed emotionally to prepare for treatment, 23%) and Emotional (Worry, about injections, 37%, Unhappiness with injection frequency, 25%, and Fear, 22%). Parent TB domains were Emotional (Worry or anxiety about treatment or treatment administration, 58% and Worry about causing pain, 42%) and Interference (Time spent preparing and administering injection, 42%, Interference with travel/vacation, 42%, and Interference with daily/family routines, 35%). The items were cognitively debriefed in a new sample (N=26: 13 children, 13 parents) and, based on findings, it was determined that the PRO version was appropriate for children aged 9–12. The final measure includes 17 items (child PRO and parent ObsRO versions) with a 12-item parent PRO module. **CONCLUSIONS:** The conceptual validity of the TB-CGHD is supported by these qualitative findings and the measure is now ready for psychometric validation.

SYSTEMIC DISORDERS/CONDITIONS – Health Care Use & Policy Studies

PSY100

TOWARDS CREATING A NATIONAL SYSTEM OF CARE FOR PATIENTS WITH ORPHAN DISEASES

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OBJECTIVES: There is no integrated approach to the problem of orphan diseases (OD) in Ukraine. Diagnosis of these illnesses is often complicated and delayed, the treatment is often ineffective because of the lack and unavailability of essential medicines and methods of treatment, the quality of life for most patients remains poor. The Law of Ukraine 'On amendments to the Basic Laws of Ukraine on Health Care on prevention and treatment of rare (orphan) diseases' has been recently issued. It governs the measures of OD prophylaxis. **METHODS:** According to the Law, in order to improve OD patient care, a work on medical and technological documents on such topics as mucopolysaccharidosis, Gaucher disease, phenylketonuria, hemophilia was launched. The development of medico-technological documents was done in a few steps. Firstly, the search for clinical guidelines was conducted in the GIN library and other sources. Secondly, the primary selection and appraisal with AGREE II instrument have been carried out. The next step was adaptation of clinical guidelines and development of clinical protocols of the medical care. It was conducted by multidisciplinary working groups including leading specialists in clinical genetics, health care managers and patients organizations. **RESULTS:** Today the clinical protocols of treatment of mucopolysaccharidosis patients, based on evidence of the effectiveness of medical technologies, pharmacotherapy and organizational principles of its provision, were approved. The documents contained modern approaches for diagnostics and treatment of mucopolysaccharidosis, which are ajusted to the health care system of Ukraine. **CONCLUSIONS:** Harmonization of treatment practices of OD in Ukraine with international best practice will bring the treatment of these states in Ukraine to a new level and provide thorough measures aimed at the development of screening and prevention programs, diagnostics and treatment, as well network collaboration among health services, educational institutions, public etc.

PSY101

MEDICAL CARE AND COSTS OF PATIENTS WITH SYSTEMIC LUPUS ERYTHEMATOSUS IN TAIWAN

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OBJECTIVES: Systemic lupus erythematosus (SLE) is a chronic autoimmune disorder that can cause multi-organ damage, leading to reduced life expectancy, lower quality of life, and high medical expenditures. Therefore, long-term and comprehensive disease management and monitoring are important. This study aimed to examine treatment pattern, quality of SLE care, and utilization and costs of medical care of SLE patients. **METHODS:** The National Health Insurance Research Database was adopted to identify patients diagnosed with SLE before 2010 through their eligibility for catastrophic illness of SLE, and examine their use and costs of health services in 2010. Logistic regression models and generalized linear models were adopted for analyses. **RESULTS:** The majority of 744 patients with SLE was female (85%), older than 40 years old (54%), and having been diagnosed with SLE for more than five years (68%). Sixty-one percent of them received corticosteroids, 59% received antimalarials, and 36% received immunosupressants. Annual tests for complete blood count, creatinine, and serum levels of complement component C3/C4 and anti-dsDNA antibodies were received by 85%, 78%, and 76% of the subjects, respectively. Forty-four percent of them received annual evaluation of cardiovascular risk, and 3% of them received influenza vaccination. Average number of outpatient visit was 33.47, and SLL-related visit was 12.82. Twenty-two percent of them were admitted to hospital during the year, and the average number of hospital admission was 0.48. The average annual medical cost was NTD105,059, and the average SLE-related medical cost was NTD29,770. Shorter time since SLE diagnosis was associated with more intense treatment, better adherence to recommendations for annual tests, and higher SLE-related costs. **CONCLUSIONS:** This study identified treatment pattern, quality of SLE care, and use and costs of patients with SLE. Disease management and monitoring need to be improved.

PSY102

THE RHEUMATOLOGIST'S PERSPECTIVE IN DIAGNOSTIC COURSE AND MANAGEMENT OF FAMILIAL MEDITERRANEAN FEVER

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OBJECTIVES: To investigate the global perspectives of Turkish Rheumatologists regarding expected FMF prevalence, undiagnosed patients ratio, arguments in diagnostic procedure and management of FMF. **METHODS:** A questionnaire consist of estimated frequency, diagnostic modalities and follow up characteristics in addition to therapeutic approach was set up. Even though a regular colchicine use, ≥1 attack / month during 6 months was accepted as colchicine resistant. All members of Turkey Society for Rheumatology from country wide (n=235) were invited to fill in the web-based questionnaire for FMF by e-mail. Totally 108 members (45.9%) answered the questionnaire. **RESULTS:** Estimated prevalence was median 0.2% (IQR25-75: 0.1-0.7) for FMF. Only 51.4±19.0 % of these patients had been diagnosed according to Rheumatologists opinion. Time to diagnosis from the first symptom was more than 3 years in 49.5% of patients. Description of typical attacks in anamnesis is the most frequent parameter in diagnosis of patients whereas in 26.7 %of patients MEFV mutations were required for diagnosis. 53.5 ±21.3 % of patients were on regular follow up and 75.7% of these follow up was with a 3-6 months period. Frequency of Colchicine resistant patients was expected in 7.2%. The use of biologic agents in the patients followed up by these physicians was expected as 3.4%. **CONCLUSIONS:** Since half of the estimated prevalence was expected as undiagnosed and patients have a 3-years delay in diagnosis, Medical and Social Activities are required to increase the awareness of FMF. Less than 5 percent of patients required biologic agents in FMF.

PSY103

IMPACT OF BLOOD TRANSFUSIONS ON HOSPITAL LENGTH OF STAY AND MORTALITY: A SINGLE-CENTER EXPERIENCE

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